

## Accelerating Medicines Partnership

The Accelerating Medicines Partnership (AMP) is a bold new venture between the National Institutes of Health (NIH), 10 biopharmaceutical companies and several non-profit organizations to transform the current model for developing new diagnostics and treatments by jointly identifying and validating promising biological targets of disease. AMP will begin with three to five year pilot projects in three disease areas: Alzheimer's disease, type 2 diabetes, and the autoimmune disorders of rheumatoid arthritis and systemic lupus erythematosus (lupus).

For each pilot, scientists from NIH and industry have developed research plans aimed at characterizing effective molecular indicators of disease called biomarkers and distinguishing biological targets most likely to respond to new therapies. The ultimate goal is to increase the number of new diagnostics and therapies for patients and reduce the time and cost of developing them.

Through this cross-sector partnership, which will be managed through the Foundation for the NIH (FNIH), NIH and industry partners are sharing expertise and resources –\$230 million – in an integrated governance structure that enables the best informed contributions to science from all participants. A critical component of the partnership is that industry partners have agreed to make the AMP data and analyses publicly accessible to the broad biomedical community. These pilot projects will set the stage for broadening AMP to other diseases and conditions

### The Opportunity

As a result of technological revolutions in genomics, proteomics, imaging, and more, researchers have been able to identify changes in genes, proteins, and other molecules that cause disease and influence disease progression. Doctors use this information to determine the presence of disease through biomarkers with the use of diagnostic tests. Biopharmaceutical companies use it to develop therapies that target these specific genes and molecules in order to interfere with their processes and modify the course of disease. These treatments are called targeted therapies. An example of a targeted therapy is the drug imatinib (Gleevec), which was designed to stop an altered enzyme produced by a fused version of two genes found in chronic myelogenous leukemia. In the last five years, researchers have identified more than 1,000 new biological changes that hold promise as biomarkers and drug targets, offering a potential revolution in diagnostics and therapeutics<sup>1</sup>.

#### AMP Partners

##### Government

- FDA
- NIH

##### Industry

- AbbVie
- Biogen Idec
- Bristol-Myers Squibb
- GlaxoSmithKline
- Johnson & Johnson
- Lilly
- Merck
- Pfizer
- Sanofi
- Takeda

##### Non-Profit Organizations

- Alzheimer's Association
- American Diabetes Association
- Foundation for the NIH
- Geoffrey Beene Foundation
- Lupus Foundation of America
- PhRMA
- Rheumatology Research Foundation
- USAgainstAlzheimer's

<sup>1</sup> Catalog of Published Genome-Wide Association Studies Database: <http://www.genome.gov/gwastudies/>

## The Challenge

While technological advances have produced a wealth of data on the biological cause of disease, moving these discoveries into treatments has been far more difficult. Not all biological insights lead to effective drug targets, and choosing the wrong target can result in failures late in the drug development process, costing time, money, and ultimately, lives. Developing a new drug – from early discovery through Food and Drug Administration (FDA) approval – takes well over a decade and has a

failure rate of more than 95 percent. As a consequence, each success costs more than \$1 billion<sup>2,3</sup>. The most expensive failures happen in late phase clinical trials, with a lack of drug efficacy currently estimated as responsible for 59 percent of Phase II failures and 52 percent of Phase III failures<sup>4</sup>. Therefore, it's essential to do a better job of pinpointing the right biological targets early in the process.

The entire biomedical research community and the public have a shared interest in compressing the timelines, reducing the costs, and increasing the success rates of new targeted therapies. Given the amount and complexity of the data, this goal will require a systematic approach in which government, academia, industry, and patient groups work collaboratively to sift through the flood of disease targets and find the ones most likely to prove responsive to treatments.

## The Impact

By optimizing the process for identifying and validating clinically relevant disease targets for drug design, AMP aims to:

### *Increase efficiency:*

- Shorter development time: accelerating the hard work of sifting through a large number of candidates to identify the best biological targets for drug development could shave months or even years off of the early stages of discovery.
- Improved prospects for success: with disease targets and biomarkers that have been validated rigorously with human data, higher confidence about efficacy should be achieved, allowing researchers to move the most promising compounds quickly into the pipeline with the expectation of fewer failures in late stage clinical trials.
- Lower costs: shorter development timeframes and fewer late-stage drug failures should reduce the cost of delivering new and effective medicines to patients.

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<sup>2</sup> B Munos. Lessons from 60 years of pharmaceutical innovation. *Nat. Rev. Drug Discov.* **8**, 959–968 (2009).

<sup>3</sup> SM Paul, DS Mytelka, CT Dunwiddie, CC Persinger, et al. How to improve R&D productivity: The pharmaceutical industry's grand challenge. *Nat. Rev. Drug Discov.* **9**, 203–214 (2010).

<sup>4</sup> J Arrowsmith, P Miller. Trial Watch: Phase II and Phase II attrition rates 2011-2012. *Nat. Rev. Drug Discov.* **12**, 569, (2013).

*Improve the process:*

- Better understanding of biological targets and identification of valid biomarkers will enable more robust clinical trials – in part by testing therapies on patients most likely to respond to them based on the molecular profiles of their disease.

*Increase the number and effectiveness of new targeted therapies:*

- Understanding the biological pathways underlying disease and the specific biological targets that can alter disease will lead to more rational drug design and better tailored therapies.
- Reducing the number of failures in Phase II and Phase III clinical trials will increase the number of new drugs developed per \$1 billion of research and development investment.
- The increase in expected returns will likely make drug development a more attractive investment.

## Governance

Steering committees for each of the three disease areas, with representation from all partners, will meet regularly to define research agendas, develop project plans, and review ongoing progress and milestones. After AMP research grants are awarded, investigators carrying out the research will be added to the disease steering committees. The steering committees will be managed by FNIH under the direction of an AMP Executive Committee comprised of representatives from NIH, participating industry leaders, FDA, and patient advocacy organizations.

## Budget: 5 years (\$229.5 Million Total Project Funding)

(\$Millions)	Total Project	Total NIH	Total Industry
<b>Alzheimer's Disease</b>	129.5	67.6	61.9
<b>Type 2 Diabetes</b>	58.4	30.4	28
<b>Rheumatoid Arthritis and Lupus</b>	41.6	20.9	20.7
<b>Total</b>	229.5	118.9	110.6